



On November 18th, 2014, a UCLA research team led by Donald Kohn, M.D., announced a breakthrough gene therapy and stem cell cure for "bubble baby" disease, or severe combined immunodefiency (SCID). Babies born with SCID lack an immune system and have no ability to fight off infections so even a minor cold could be deadly. The team's treatment approach cured all 18 children who participated in the trial.

CIRM has awarded over \$13 million to Kohn's team to support a clinical trial that will apply this treatment strategy to cure sickle cell disease. The trial is set to begin in early 2015.

 $\textbf{Source URL:} \ http://www.cirm.ca.gov/our-progress/video/stem-cell-cure-bubble-baby-disease-scid-pioneered-uclas-don-kohn with the control of the contro$